Louisiana Medicaid
Golodirsen (Vyondys 53®)

The *Louisiana Uniform Prescription Drug Prior Authorization Form* should be utilized to request clinical authorization for golodirsen (Vyondys 53®).

Additional Point-of-Sale edits may apply.

Vyondys 53® is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Vyondys 53®. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Approval Criteria

- The following is true and is **stated on the request** - The recipient has a genetically-confirmed diagnosis of Duchenne muscular dystrophy [ICD-10-CM code G71.0] and lab testing confirms that the mutation of the dystrophin (DMD) gene is amenable to exon 53 skipping; **AND**
- The recipient is at least 6 years of age, but not older than 13 years of age at initiation of therapy; **AND**
- The recipient has been on a stable dose of corticosteroids for at least 6 months prior to the date of the request. Each medication and date range of treatment must be **listed on the request**; **AND**
- Vyondys 53® is prescribed by, or in consultation with, a neurologist experienced in the diagnosis and treatment of DMD; **AND**
- The recipient has functional status that can be preserved as demonstrated by a valid measuring tool (name and result of the valid measuring tool is **stated on the request)**; **AND**
- By submitting the authorization request, the prescriber attests to the following:
    - The prescribed dose does not exceed 30mg/kg once weekly; **AND**
    - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning(s), Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
    - All laboratory testing and clinical monitoring parameters are completed as instructed in the prescribing information and will be repeated as recommended; **AND**
    - The recipient has no concomitant drug therapies [including other RNA antisense agent(s) like eteplirsen, or any other gene therapy] or disease states that would limit the use of the requested medication and will not be receiving the requested medication in combination with any medication that is contraindicated or not recommended per FDA labeling**.**

Reauthorization Criteria

- The recipient started treatment before 14 years of age; **AND**
- Vyondys 53® is prescribed by, or in consultation with, a neurologist experienced in the diagnosis and treatment of DMD; **AND**
- The following are true and the prescriber **states both on the request**:
    - **There has been a positive response to therapy**; **AND**
    - Functional status is reassessed with the same valid measuring tool, and the results show stable or improved functional status from baseline (**baseline and reassessment results** are stated on the request); **AND**
- By submitting the reauthorization request, the prescriber attests to the following:
    - The prescribed dose does not exceed 30mg/kg once weekly; **AND**
    - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning(s), Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
    - All laboratory testing and clinical monitoring parameters are completed as instructed in the prescribing information and will be repeated as recommended; **AND**
    - The recipient has no concomitant drug therapies [including other RNA antisense agent(s) like eteplirsen, or any other gene therapy] or disease states that would limit the use of the requested medication and will not be receiving the requested medication in combination with any medication that is contraindicated or not recommended per FDA labeling**.**

Duration of authorization approval, both initial and reauthorization: 6 months

References

National Institute of Health, U.S. Department of Health & Human Services. Duchenne muscular dystrophy. (2017, September 28). Retrieved from https://rarediseases.info.nih.gov/diseases/6291/duchenne-muscular-dystrophy

Vyondys 53 (golodirsen) [package insert]. Cambridge, MA: Sarepta Therapeutics Inc; August 2020. https://dailymed.nlm.nih.gov/dailymed/fda/fdaDrugXsl.cfm?setid=35c227d1-5b24-44b0-b5d3-f0f6b1c46bd5&type=display

Revision	Date
Policy created.	January 2020
Policy implemented.	May 2020
Removed dystrophin level requirement and updated package insert	September 2020